



4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2018-N-4609]

Issuance of Priority Review Voucher; Rare Pediatric Disease Product

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

SUMMARY: The Food and Drug Administration (FDA) is announcing the issuance of a priority review voucher to the sponsor of a rare pediatric disease product application. The Federal Food, Drug, and Cosmetic Act (FD&C Act), as amended by the Food and Drug Administration Safety and Innovation Act (FDASIA), authorizes FDA to award priority review vouchers to sponsors of approved rare pediatric disease product applications that meet certain criteria. FDA is required to publish notice of the award of the priority review voucher. FDA has determined that ZOLGENSMA (onasemnogene abeparvovec-xioi), manufactured by AveXis, Inc., meets the criteria for a priority review voucher.

FOR FURTHER INFORMATION CONTACT: Shruti Modi, Center for Biologics Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 71, Rm. 7301, Silver Spring, MD 20993-0002, 240-402-7911.

SUPPLEMENTARY INFORMATION: FDA is announcing the issuance of a priority review voucher to the sponsor of an approved rare pediatric disease product application. Under section 529 of the FD&C Act (21 U.S.C. 360ff), which was added by FDASIA, FDA will award priority review vouchers to sponsors of approved rare pediatric disease product applications that meet

certain criteria upon approval of those applications. FDA has determined that ZOLGENSMA (onasemnogene abeparvovec-xioi), manufactured by AveXis, Inc., meets the criteria for a priority review voucher. ZOLGENSMA (onasemnogene abeparvovec-xioi) is indicated for the treatment of pediatric patients less than 2 years of age with spinal muscular atrophy with biallelic mutations in the survival motor neuron 1 gene.

For further information about the Rare Pediatric Disease Priority Review Voucher Program and for a link to the full text of section 529 of the FD&C Act, go to <https://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/RarePediatricDiseasePriorityVoucherProgram/default.htm>. For further information about ZOLGENSMA (onasemnogene abeparvovec-xioi), go to the Center for Biologics Evaluation and Research Cellular and Gene Therapy Products website at <https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products>.

Dated: June 18, 2019.

Lowell J. Schiller,

Principal Associate Commissioner for Policy.

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